



4160-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-N-2013-1328]

Sickle Cell Disease Public Meeting on Patient-Focused Drug Development

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public meeting; request for comments.

SUMMARY: The Food and Drug Administration (FDA) is announcing a public meeting and an opportunity for public comment on Patient-Focused Drug Development for sickle cell disease.

Patient-Focused Drug Development is part of FDA's performance commitments in the fifth authorization of the Prescription Drug User Fee Act (PDUFA V). The public meeting is intended to allow FDA to obtain patients' perspectives on the impact of sickle cell disease on daily life and on available therapies for sickle cell disease.

DATES: The public meeting will be held on February 7, 2014; from 10 a.m. to 4 p.m.

Registration to attend the meeting must be received by January 27, 2014. See the

SUPPLEMENTARY INFORMATION section for information on how to register for the meeting. Submit electronic or written comments by April 8, 2014.

ADDRESSES: The meeting will be held at the FDA White Oak Campus, 10903 New Hampshire Ave., Bldg. 31 Conference Center, in Sections B and C of the Great Room (rm. 1503), Silver Spring, MD 20993. Entrance for the public meeting participants is through Building 1, where routine security check procedures will be performed. For more information on parking and security procedures, please refer to

<http://www.fda.gov/AboutFDA/WorkingatFDA/BuildingsandFacilities/WhiteOakCampusInformation/ucm241740.htm>.

Submit electronic comments to [www.regulations.gov](http://www.regulations.gov). Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number found in brackets in the heading of this document.

FDA will post the agenda approximately 5 days before the meeting at:

<http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm370867.htm>.

FOR FURTHER INFORMATION CONTACT: Graham Thompson, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 51, rm. 1199, Silver Spring, MD 20993, 301-796-5003, FAX: 301-847-8443, email:

[Graham.Thompson@fda.hhs.gov](mailto:Graham.Thompson@fda.hhs.gov).

#### SUPPLEMENTARY INFORMATION:

##### I. Background on Patient-Focused Drug Development

FDA has selected sickle cell disease to be the focus of a meeting under Patient-Focused Drug Development, an initiative that involves obtaining a better understanding of patients' perspectives on the severity of the disease and the available therapies for the condition. Patient-Focused Drug Development is being conducted to fulfill FDA's performance commitments made as part of the authorization of PDUFA V under Title I of the Food and Drug Safety and Innovation Act (Pub. L. 112-144). The full set of performance commitments is available on the FDA Web site at

<http://www.fda.gov/downloads/forindustry/userfees/prescriptiondruguserfee/ucm270412.pdf>.

FDA has committed to obtain the patient perspective in 20 disease areas during the course of PDUFA V. For each disease area, the Agency will conduct a public meeting to discuss the disease and its impact on patients' daily lives, the types of treatment benefit that matter most to patients, and patients' perspectives on available therapies for sickle cell disease. These meetings will include participation of FDA review divisions, the relevant patient community, and other interested stakeholders.

On April 11, 2013, FDA published a notice (78 FR 21613) in the Federal Register announcing the disease areas for meetings in fiscal years (FYs) 2013 through 2015, the first 3 years of the 5-year PDUFA V timeframe. To develop the list of disease areas, the Agency used several criteria that were outlined in the April 2013 notice. The Agency obtained public comment on these criteria and potential disease areas through a notice for public comment published in the Federal Register on September 24, 2012 (77 FR 58849), and through a public meeting held on October 25, 2012. In selecting the disease areas, FDA carefully considered the public comments received and the perspectives of its review divisions. By the end of FY 2015, FDA will initiate another public process for determining the disease areas for FYs 2016 and 2017. More information, including the list of disease areas and a general schedule of meetings, is posted on FDA's Web site at

<http://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm326192.htm>.

## II. Public Meeting Information

### A. Purpose and Scope of the Meeting

As part of Patient-Focused Drug Development, FDA will obtain patient and patient stakeholder input on sickle cell disease and on current approaches to treatment. Approximately 100,000 people in the United States, and millions of people worldwide, have sickle cell disease.

Sickle cell disease is an inherited red blood cell disorder resulting from a mutation in the beta globin gene. Red blood cells are more prone to an abnormal shape and rigidity, causing multi-organ damage over time. Some of the effects of sickle cell disease are painful crises, increased risk of infections, stroke, pulmonary hypertension, acute chest syndrome, recurrent priapism, gallstones, and kidney dysfunction.

Therapies to prevent the complications of sickle cell disease are limited and can include prescription medications and blood transfusions. Bone marrow transplantation is an option for some patients. Other therapies, such as pain medications, antibiotics, supplemental oxygen, and vitamin supplements, are used to manage specific health effects of the disease. New approaches to treating sickle cell disease or preventing its complications are being explored, including new medications, advances in transplantation, and gene therapies. FDA is interested in obtaining a better understanding of patients' perspectives on sickle cell disease, including the symptoms that matter most to patients, limitations to current treatment approaches, opportunities for new treatment approaches, and specific considerations regarding sickle cell disease in pediatric patients.

The questions that will be asked of patients and patient stakeholders at the meeting are listed in this section, organized by topic. For each topic, a brief patient panel discussion will begin the dialogue, followed by a facilitated discussion inviting comments from other patient and patient stakeholder participants. In addition to input generated through this public meeting, FDA is interested in receiving patient input addressing these questions through written comments that can be submitted to the public docket (see ADDRESSES). When submitting comments to the docket, please provide some context to your comment by indicating whether you are an adolescent or young adult, or older adult. If you are commenting on behalf of a child or other

loved one who has sickle cell disease, please indicate that and answer the following questions as much as possible from the patient's perspective.

Topic 1: The effects of sickle cell disease that matter most to you

1. Of all of the ways that sickle cell disease affects your health, which one to three effects have the greatest impact on your life? (Examples may include pain crises, breathing problems, difficulty concentrating, tiredness, infections, and others.)
2. How does sickle cell disease affect your life on an "average" day?
  - a. Are there activities that you cannot do at all or as well as you would like on these "average" days? Please describe, using specific examples. (Examples may include sleeping through the night, concentrating at work or at school, participating in physical activities, and others.)
3. How does sickle cell disease affect your life on the "worst" days, such as days when you have a pain crisis or have to be hospitalized for some reason?
  - a. Are there activities that you cannot do at all or as well as you would like on these "worst" days? Please describe, using specific examples.
4. What worries you most about how sickle cell disease could affect your health in the future?
5. What specific concerns do you have about sickle cell disease:
  - a. In infants and young children?
  - b. In adolescents and young adults?
  - c. In older adults?

Topic 2: Perspectives on treatments for sickle cell disease

1. Are you currently using any prescription medicines or medical treatments to prevent or treat any negative effects of your sickle cell disease? Please describe these treatments, which may include blood transfusions, supplemental oxygen and prescription medications such as hydroxyurea, antibiotics, pain medications, and others.
  - a. How well do these treatments work for you? For example, how well do they reduce your number of pain crises, hospitalizations, or strokes? How well do they help you manage your pain, breathing difficulties, or other health effects?
  - b. What are the biggest problems with these treatments? (Examples may include side effects of medicine, going to the hospital for treatment, frequent blood tests, etc.)  
How do these problems affect your daily life?
2. Besides prescription medications, what else do you do to prevent or treat any negative effects of your sickle cell disease? Please describe any medications purchased at a store without a prescription, home remedies, diet changes, massages, or other therapies.
  - a. What specific parts of your sickle cell disease do these treatments address?
  - b. How well do these treatments work for you?
  - c. What are the biggest problems with these treatments?
3. What parts of your sickle cell disease do your current treatments not treat at all or not as well as you would like?
4. Assuming that there is no cure for sickle cell disease, what specific things would you look for in an ideal treatment?
5. If you had the opportunity to consider participating in a clinical trial studying experimental treatments for sickle cell disease, what things would you consider when deciding whether or not to participate? Examples may include how severe your sickle

cell disease is, how well current treatments are working for you, your concern about serious risks, and other things.

B. Meeting Attendance and/or Participation

If you wish to attend this meeting, visit <https://patientfocusedsicklecell.eventbrite.com>. Please register by January 27, 2014. Those who are unable to attend the meeting in person can register to participate in a live Webcast of the meeting. You will be asked to indicate in your registration whether you plan to attend in person or via the Webcast. Your registration should also contain your complete contact information, including name, title, affiliation, address, email address, and phone number.

Seating will be limited, so early registration is recommended. Registration is free and will be on a first-come, first-served basis. However, FDA may limit the number of participants from each organization based on space limitations. Registrants will receive confirmation once they have been accepted. Onsite registration on the day of the meeting will be based on space availability. If you need special accommodations because of disability, please contact Graham Thompson (see FOR FURTHER INFORMATION CONTACT) at least 7 days before the meeting.

Patients who are interested in presenting comments as part of the initial panel discussions will be asked to indicate in their registration which topic(s) they wish to address. They will also be asked to send a brief summary of responses to the topic questions to [PatientFocused@fda.hhs.gov](mailto:PatientFocused@fda.hhs.gov). Panelists will be notified of their selection soon after the close of registration on January 27, 2014. FDA will try to accommodate all patients and patient stakeholders who wish to speak, either through the panel discussion or audience participation; however, the duration of comments may be limited by time constraints.

Interested members of the public, including those who attend the meeting in person or through the Webcast, are invited to provide electronic or written responses to the questions pertaining to Topics 1 and 2 to the public docket (see ADDRESSES). Comments may be submitted until April 8, 2014.



Dated: October 31, 2013.

Leslie Kux,

Assistant Commissioner for Policy.

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